CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Onpattro (patisiran)
BILLING CODE	J0222
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home/Office/Outpatient
STATUS	Prior Authorization Required

Onpattro contains a transthyretin-directed small interfering RNA (siRNA) and is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults. It is an RNA interference (RNAi) drug that causes degradation of mutant and wild-type TTR mRNA, which results in a reduction of serum TTR protein and TTR protein deposits in tissues by targeting the liver where TTR protein is synthesized. In the APOLLO clinical trial, changes from baseline to Month 18 on both the mNIS+7 (primary endpoint) and the Norfolk QoL-DN significantly favored Onpattro, as well as changes in modified body mass index (mBMI) and gait speed (10-meter walk test). Changes were evident at 9 months.
hATTR is a rare and progressive inherited disorder where misfolded TTR accumulates as amyloid fibrils in the body. In polyneuropathy of hATTR (hATTR-PN), these fibrils deposit in the peripheral nerves which leads to pain, muscle weakness, and autonomic dysfunction. Onpattro is administered by a healthcare professional every 3 weeks via IV infusion.

Onpattro (patisiran) will be considered for coverage when the following criteria are met:

Hereditary Transthyretin Amyloidosis (hATTR Amyloidosis): Polyneuropathy

For **initial** authorization:

1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist; AND
3. Member has a diagnosis of hATTR amyloidosis with documentation of a transthyretin (TTR) mutation confirmed by genetic testing; AND
4. Member has signs/symptoms of polyneuropathy; AND
5. Member has a polyneuropathy disability (PND) score of IIIb or less (i.e., member is not wheelchair-bound or bedridden); AND
6. Member has NOT had a liver transplant; AND
7. Onpattro is NOT being used in combination with another hATTR drug (e.g., Amvuttra, Tegsedi, Vyndaqel, Vyndamax).
8. **Dosage allowed/Quantity limit:** For members weighting less than 100 kg: 0.3 mg/kg every 3 weeks. For members weighing 100 kg or more, the recommended dosage is 30 mg every 3 weeks. (QL: 3 vials per 21 days)

If all the above requirements are met, the medication will be approved for 9 months.

For **reauthorization**:

1. Chart notes must include documentation of positive clinical response to therapy such as improvements in neuropathy impairment, gait speed, nutritional status, disability, or quality of life compared to baseline.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Onpattro (patisiran) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
08/05/2019	New policy for Onpattro created.
07/02/2020	Simplified diagnostic requirement of hATTR to just any method of confirmation by chart notes. Separated genetic testing and FAP staging into their own mandatory requirements. Expanded prescriber to include physicians who specialize in treating amyloidosis.
08/02/2022	Transferred to new template. Updated and added references. Removed other specialists except neurology. Removed exclusions except liver transplant. Replaced FAP staging with PND score. Added QL. Increased initial auth duration from 6 mo to 9 mo. Edited renewal criteria to be consistent with Amvuttra.

References:

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6. Sekijima Y. Hereditary Transthyretin Amyloidosis. 2001 Nov 5 [Updated 2021 Jun 17]. In: Adam MP, Mirzaa GM, Pagon RA, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2022. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1194/
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8. Adams D, Ando Y, Beirão JM, et al. Expert consensus recommendations to improve diagnosis of ATTR amyloidosis with polyneuropathy. *J Neurol*. 2021;268(6):2109-2122. doi:10.1007/s00415-019-09688-0
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